

CLAIMS

WHAT IS CLAIMED:

5 1. A method for delivering a nucleic acid molecule to a location in an animal, wherein said location is inaccessible to direct protein delivery, comprising the step of administering a positively charged liposome to said animal, wherein said positively charged liposome is associated with said nucleic acid molecule, wherein said nucleic acid molecule is in operable association with a promoter.

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15 2. The method of claim 1, wherein said nucleic acid molecule encodes human α_1 antitrypsin.

 3. The method of claim 1, wherein said nucleic acid molecule encodes prostaglandin synthase.

20 4. The method of claim 1, wherein said location is selected from the group consisting of an endothelial lung cell, a smooth muscle cells adjacent to said endothelial lung cell, and lung parenchyma.

25 5. The method of claim 1, wherein said location is selected from the group consisting of a liver cell, a muscle cell, an osteogenic cell, synoviocyte, and a lung cell.

6. The method of claim 1, wherein said animal is a mammal.

7. The method of claim 6, wherein said mammal is a human.

8. The method of claim 1, wherein said positively charged liposome is Lipofectin™.

9. The method of claim 1, wherein said nucleic acid sequence encodes a therapeutically effective protein and wherein said method further comprises expressing said nucleic acid sequence to provide said protein to said location.

10. The method of claims 9, wherein said protein is an antiprotease.

11. The method of claim 1, wherein at least 10% of said nucleic acid that is administered is delivered to said location.

12. The method of claim 11, wherein at least 50% of said nucleic acid that is administered is delivered to said location.

13. The method of claim 12, wherein at least 90% of said nucleic acid that is administered is delivered to said location.

14. A method for preventing or treating an animal having a disorder, wherein at least one symptom associated with said disorder is caused at least in part by an insufficient amount or form of protein in a particular location of said animal, wherein said method comprises the step of delivering a gene encoding said protein to said location and expressing said gene.

15. The method of claim 14, wherein said gene encodes human α_1 antitrypsin.

16. The method of claim 14, wherein said gene encodes prostaglandin synthase.

17. The method of claim 14, wherein said location is selected from the group consisting of an endothelial lung cell, a smooth muscle cells adjacent to said endothelial lung cell, and lung parenchyma.

18. The method of claim 14, wherein said location is selected from the group consisting of a liver cell, a muscle cell, an osteogenic cell, synoviocyte, and a lung cell.

19. The method of claim 14, wherein said animal is a mammal.

20. The method of claim 19, wherein said mammal is a human.

21. The method of claim 14, wherein said positively charged liposome is Lipofectin.

22. The method of claim 14, wherein said gene encodes a therapeutically effective protein.

23. The method of claims 22, wherein said protein is an antiprotease.

24. The method of claim 14, wherein at least 10% of said nucleic acid that is administered is delivered to said location.

25. The method of claim 24, wherein at least 50% of said nucleic acid that is administered is delivered to said location.

26. The method of claim 25, wherein at least 90% of said nucleic acid that is administered is delivered to said location.

27. A method for delivering a nucleic acid molecule to a location in an animal, comprising the step of administering a positively charged liposome to said animal, wherein said positively charged liposome is associated with said nucleic acid molecule, wherein said nucleic acid molecule is in operable association with a promoter, wherein delivery of said gene is capable of producing a therapeutic response but direct delivery of

the protein encoded by said gene does not produce a therapeutic response.

5 28. The method of claim 27 wherein delivery of said gene produces a therapeutic response even when delivered at a 20-fold lower serum concentration than said protein which does not produce a therapeutic response when delivered directly as a protein.

10 29. The method of claim 27, wherein said therapeutic response is elimination of an endotoxin induced increase in pulmonary vascular resistance.

15 30. A method of producing an elevated therapeutic response relative to the therapeutic response created by direct delivery of a protein comprising the step of delivering a nucleic acid molecule encoding said protein.

20 31. The method of claim 30, wherein the therapeutic response created by direct delivery of said protein is non-existent or immeasurable.

25 32. The method of claim 30, wherein said protein is an antiprotease.

33. The method of claim 30, wherein said enhanced therapeutic response is created in a patient suffering from a disorder selected from the group consisting of

adult respiratory distress syndrome, cystic fibrosis, respiratory syncytial virus infection, interstitial lung disease, and chronic obstructive pulmonary disease.

5 34. The method of claim 1, wherein said method results in generation of the protein encoded by the nucleic acid molecule, and wherein said protein is in a location which is inaccessible to direct protein deliver.

10 35. The method of claim 5 or 18 wherein said location is a lung epithelial cell.